2017 Annual Report

The CIRM 2017 Annual Report details how California's Stem Cell Agency is transforming medicine, lives and futures by accelerating stem cell treatments to patients with unmet medical needs.

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CIRM
CALIFORNIA'S STEM CELL AGENCY
2017 ANNUAL REPORT
TRANSFORMING medicine lives the future

MISSION

Accelerate stem cell treatments to patients with unmet medical needs

With our help, good ideas have the power to transform medicine, lives and the future.

It takes commitment and courage to challenge what we've done before, to push ourselves harder, to always get better to fulfill our mission.

Because every moment counts.

At CIRM, we never forget that we were created by the people of California when they approved Proposition 71 in 2004, authorizing $3 billion to fund stem cell research in California.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

CIRM is focused on achieving its mission through the timely and efficient funding of high-quality stem cell projects and accelerating delivery of resulting treatments and cures to patients in need.

For more information go to **www.cirm.ca.gov**

A NEW LEADER AT THE HELM

Identifying the right person to lead an organization can be very challenging.

However, when Randy Mills announced he was stepping down as CIRM's President and CEO in May this year, the decision about who should follow him was simple.

Maria Millan, M.D., has been a key member of our team since joining CIRM in 2012.

Maria was the driving force behind CIRM's Alpha Stem Cell Clinic Network and helped shape our strategic plan, which has been—and will continue to be—our agency's road map for the next few years. As head of our Therapeutics' team, Maria was responsible for spearheading our search for high-quality clinical trial applicants. With her background as a transplant surgeon, researcher and biotech executive, she has been relentless in her commitment to the health of patients. Maria was a natural choice to succeed Randy.

Our CIRM Board believes Maria is the right person, at the right time, with the right skills to take us to the next level—and drive the future of the field.

Besides bringing an "all-in" attitude to everything she does, Maria also represents continuity at CIRM, helping us stay on-course to meet our mission to accelerate stem cell treatments to patients with unmet medical needs—and with the unmistakable urgency this work demands. Every single day.

Yours in the best of health,

Jonathon Thomas

Jonathan Thomas, Ph.D., J.D.
CHAIRMAN, INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE

BREAKING NEW GROUND

I am honored and fortunate to take on the role of CIRM's new President and CEO during such a momentous time in CIRM's history and in the stem cell and regenerative medicine field.

We continue to break new ground.

In 2017, CIRM programs were the first to obtain the FDA's freshly minted Regenerative Medicine Advanced Therapy (RMAT) designation. This expedited pathway was made possible by the bipartisan 21st Century Cures Act that recognizes the importance of stem cell research by putting resources in place to accelerate the field.

In 2017, the FDA granted marketing approval for the first two gene-modified cell-therapy products in the U.S. It was a long road for these programs and many potential cell and regenerative cures are yet to be developed. CIRM continues to play a crucial role in this journey. Through funding and active partnership, CIRM has delivered a clear and predictable pathway for discoveries to be developed into therapies. Cell therapy is no longer theoretical or an untethered hope.

If not for CIRM, many programs currently in clinical trials to address debilitating and fatal medical conditions might have stalled or have been discontinued due to lack of funding. As a result, we are seeing more partnerships and follow-on industry investment—almost $390 million this year—to advance CIRM-funded programs.

By investing when others are not yet ready to do so, CIRM's partnership enables researchers to develop a value proposition that attracts follow-on investors and industry partnerships.

Our streamlined application and funding process has enabled us to build a robust stem cell portfolio that is second to none. The value we bring continues to grow and gain significant attention as the field continues to expand.

I am pleased to report that we are on target to achieve and, in many cases, exceed our ambitious five-year strategic goals. At CIRM, we are driven by our unwavering commitment to patients to accelerate the development of stem cell treatments and to boldly invest in the future of medicine.

Sincerely,

Maria T. Millan

Maria T. Millan, M.D.
PRESIDENT AND CHIEF EXECUTIVE OFFICER

Learn More:
CIRM Leadership
CIRM ICOC Board Members

Accelerating Cures

Innovation drives transformation

This is why CIRM supports research at every level: from the most fundamental to the final stages before the U.S. Food and Drug Administration (FDA) approves it for use. 2017 investment in our five programs includes the following:

INFRASTRUCTURE	EDUCATION	DISCOVERY	TRANSLATION	CLINICAL
$16 MILLION	**$1 MILLION**	**$45 MILLION**	**$24 MILLION**	**$213 MILLION**
CIRM funded the expansion of the Alpha Stem Cell Clinics Network by funding two additional Alpha Clinics at UC Davis and UC San Francisco.	CIRM supported the SPARK and Bridges programs to train the next generation of stem cell scientists and hosted conferences to help share knowledge and advance the field.	We support and invest in early-stage research that explores new and groundbreaking stem cell treatments and technologies.	CIRM helps the best discovery-level research advance to the next level, by establishing, supporting and testing the foundational work required for clinical trial applications.	We are building a world-class therapeutics portfolio, and supporting each project with Clinical Advisory Panels and other resources to increase chances of success.

Imagine a life that's far shorter than it should be. Full of trips to the emergency room, extended hospital stays—and the challenges and complications of disease that can't be cured by modern medicine.

For many patients, this is their reality. But at CIRM, we want to change that. In 2016, we showed that a cure is possible. In 2017, we continued our tenacious commitment to our mission by:

- **investing in research, long before pharmaceutical companies or venture capitalists show interest;**
- **guiding and advising our partners, so together we can improve a project's chances of success; and**
- **working with patient advocates and other stakeholders to find cures.**

Because if it's up to anyone, it's up to us. Because cell-based therapies *work*.
And that's why we do what we do.

Our Impact

Accelerating stem cell treatments to patients

Because every moment counts

43 TOTAL CLINICAL TRIALS

703 TOTAL PATIENTS ENROLLED IN CIRM-FUNDED CLINICAL TRIALS

67 CLINICAL ADVISORY PANELS CONVENED IN 2017

806 PROJECTS FUNDED SINCE CIRM'S INCEPTION

25% OF ALL RMAT DESIGNATIONS ARE CIRM PROGRAMS

1,189 STEM CELL CHAMPIONS (PEOPLE WHO HAVE SIGNED UP TO BE SUPPORTERS OF—AND ADVOCATES FOR—STEM CELL RESEARCH AROUND THE U.S.)

16 NEW CLINICAL TRIALS IN 2017 ADDRESSING DEVASTATING DISEASES FOR WHICH THERE CURRENTLY ARE NO KNOWN CURES. THESE TRIALS HAVE THE POTENTIAL TO CHANGE THE LANDSCAPE OF MEDICINE AND THE FUTURE OF THOSE WHO SUFFER FROM THESE DEBILITATING CONDITIONS.

- ACUTE MYELOID LEUKEMIA (2 TRIALS)
- ALPHA THALASSEMIA MAJOR
- ALS-LOU GEHRIG'S DISEASE (2 TRIALS)
- BRAIN CANCER-GLIOMA (CAR-T CELLS)
- CHRONIC LYMPHOCYTIC LEUKEMIA (CLL)
- BONE MARROW TRANSPLANTS (2 TRIALS)
- KIDNEY DISEASE
- MULTIPLE MYELOMA
- OSTEOARTHRITIS
- SEVERE COMBINED IMMUNODEFICIENCY (SCID)
- STROKE
- TYPE 1 DIABETES (2 TRIALS)

8 NEW PARTNERSHIP EVENTS IN 2017 (LICENSES, OPTIONS, ADDITIONAL FUNDING)

2017: A TURNING POINT FOR MEDICINE

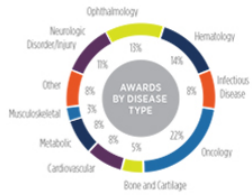
New cell-based therapies are creating a new foundation for treating and curing debilitating and deadly diseases that just a few short years ago were considered incurable.

FASTER ACCESS TO PROMISING TREATMENTS

Through the 21st Century Cures Act, the FDA introduced new procedures in 2017, such as the Regenerative Medicine Advanced Therapy (RMAT) designation, to help speed up access to the most promising therapies—progress in line with CIRM's goals.

In 2017, the FDA granted 12 projects RMAT status. Six are connected to CIRM, including three CIRM-funded clinical trials:

- **Humacyte,** creating stem cell-based blood vessels for people getting dialysis for kidney disease;
- **jCyte,** using stem cells to repair and replace the cells in the eye attacked by retinitis pigmentosa, a vision-destroying disease; and
- **Asterias,** using stem cells to repair damage caused by a spinal cord injury.



CLINICAL DEVELOPMENT PORTFOLIO

Our clinical development portfolio features 47 programs that use diverse technologies to address a wide range of diseases. CIRM's portfolio is categorized in two broad groups:

Clinical Stage Programs: 38 active programs aiming to complete a Phase 1 through Phase 3 trial in our clinic.

Investigational New Drug Filing (IND) Programs: Nine programs for which we aim to file an IND with the FDA; the last step necessary before starting a clinical trial.

CLINICAL TRIALS: A Snapshot

With a growing number of clinical trials to track—and more on the way—we needed a new tool to make it easier to see at a glance those trials we are funding, and all the key details of each program.

Our Clinical Trials Dashboard at www.CIRM.ca.gov lets you sort each trial by disease type, researcher, company or institution, and phase, as well as how many patients are to be enrolled. It also includes links to the www.clinicaltrials.gov website, with details about patient eligibility and how to apply.

Our goal is to make it as easy as possible to find the information you need.

Learn More:
CIRM Clinical Trial Dashboard

Increased Industry Engagement

CIRM's early funding in groundbreaking research and therapies has spurred industry investment to further support these projects. 2017 was a watershed year for industry engagement in CIRM-funded projects.

2017 PARTNERSHIP EVENTS: LICENSES, OPTIONS & ADDITIONAL FUNDING

CIRM's initial investment in programs and infrastructure has helped researchers, companies and institutions attract additional support.

	DISEASE	INDUSTRY PARTNER	2017 FUNDING
1	ADENOSINE DEAMINASE-DEFICIENT SEVERE COMBINED IMMUNODEFICIENCY	ORCHARD THERAPEUTICS	$110,000,000
2	X-LINKED CHRONIC GRANULOMATOUS DISEASE	ORCHARD THERAPEUTICS	NOT DISCLOSED
3	ACUTE MYELOID LEUKEMIA	FORTY SEVEN, INC.	$75,000,000
4	PEDIATRIC GENETIC DISORDER	AVROBIO, INC.	NOT DISCLOSED
5	HIV/AIDS	CSL BEHRING	$91,000,000
6	CHRONIC LYMPHOCYTIC LEUKEMIA	ONCTERNAL, INC.	$18,600,000
7	BRAIN CANCER	MUSTANG BIO, INC.	$94,500,000
8	AGE-RELATED MACULAR DEGENERATION	SANTEN PHARMACEUTICAL	NOT DISCLOSED
	TOTAL		$388,900,000 +

"CIRM has funded the full pipeline of our work on cardiac regeneration—from basic discoveries, all the way to preclinical studies. As a result of their support, we established Tenaya Therapeutics, a local start-up company that launched with $50 million in Series A investment and aims to tackle heart failure."

DEEPAK SRIVASTAVA, M.D.
President, Gladstone Institutes

LEVERAGED FUNDS TO DATE:

$1.9 BILLION

LEVERAGED FUNDING FALLS INTO 3 MAIN CATEGORIES:

CO-FUNDING — $911 MILLION
Funding from institutions, industry or investors who join with CIRM to fund a specific project at the outset. CIRM and partner funding is concurrent.

PARTNERSHIP EVENTS — $582 MILLION
Support committed by partners independent of CIRM funding to help advance a project.

ADDITIONAL FUNDING LEVERAGE — $395 MILLION
Any additional funding that a Principal Investigator can attract to a project because of CIRM funding.

Cystinosis is a rare disease that usually strikes children before they are two years old and can lead to end-stage kidney failure before their 10th birthday. Current treatments are limited, which is why the CIRM Board approved $5.2 million for research that holds the possibility of a safe, effective, one-time treatment.

Cystinosis is caused by a genetic mutation that allows an amino acid (cysteine) to build up in and damage the kidneys, eyes, liver, muscles, pancreas and brain of children and adults. There is an FDA-approved therapy, cysteamine, but this only delays progression of the disease. It has severe side effects—people taking it still require kidney transplants and may develop diabetes, neuromuscular disorders and hypothyroidism.

University of California, San Diego, researcher Stephanie Cherqui, Ph.D., and her team believe there is a better approach. Their goal is to take blood stem cells from people with cystinosis, genetically modify them to remove the mutation that causes the disease, then return them to the patient. The hope is that the modified blood stem cells will create a new, healthy, blood system free of the disease.

Even though this is an early-stage project, it is partnered with AVROBIO, a company that specializes in rare diseases—an example of early industry engagement for CIRM-funded projects.

"CIRM's support has been crucial in advancing the stem cell gene therapy approach to cystinosis. CIRM hasn't just provided critical funding but advisory support as well. Both are crucial for the success of such a project."

STEPHANIE CHERQUI, Ph.D.
Associate Professor,
University of California, San Diego

RESEARCHER
TEJAL DESAI, Ph.D.
UNIVERSITY OF CALIFORNIA, SAN FRANCISCO

NEW DIABETES TREATMENT

Every great scientific discovery begins with someone asking the question, "I wonder if...?"

That's why CIRM supports discovery: the fundamental medical research that unearths vital information that could lead to lifesaving therapies.

Leading a CIRM-funded research project to develop such a therapy for type 1 diabetes is Tejal Desai, Ph.D.—an award-winning bioengineer and accomplished educator and administrator at University of California, San Francisco.

Dr. Desai says support for discovery research is critical to advancing science. Diabetes is a huge unmet medical need. Solving it will take a multipronged solution that brings together scientists and engineers.

"This is the right time and place to tackle diabetes, and CIRM has allowed investigators with different areas of expertise to come together. Ultimately, this merging of ideas is what will make us successful."

Learn More:

STRONGER, FASTER, BETTER *operational excellence*

At CIRM, we're always looking for ways to improve so we're faster, smarter, and more efficient and effective.

Speeding up the way we work has produced some impressive results. We have lowered the amount we spend per application while increasing the speed with which those applications move through our review process. At the same time we have maintained the quality of the programs we are funding, which is reflected in the fact that 75 percent of them are hitting operational milestones on time.

OPERATIONAL ACHIEVEMENTS INCLUDE:

- Executing Strategic Plan, ahead of schedule
- Increased programs in clinical trials
- Strengthened relationships with investors and industry
- Lowered operating costs
- Expedited grant funding

75% MORE HIGH-QUALITY PROGRAMS FUNDED

82% SUCCESS CLINICAL MILESTONES

2.5X EXPANSION OF CLINICAL TRIAL PORTFOLIO

82% LESS TIME TO APPROVAL

33% MORE APPLICATIONS REVIEWED

57% LOWER COST PER APPLICATION

75% KEY MILESTONES ACHIEVED ON-TIME

GWG MEMBER

MARK NOBLE, Ph.D.
UNIVERSITY OF ROCHESTER

PIONEERING A NEW FUTURE

Mark Noble, Ph.D., is a pioneer in stem cell research and the Director of the University of Rochester Stem Cell and Regenerative Medicine Institute in New York.

Since 2011, Dr. Noble has been a member of CIRM's Grants Working Group (GWG), the panel of independent scientific experts CIRM relies on to review applications for funding and decide which are the best.

"This is the most exciting review panel," Dr. Noble says. "What sets it apart is that CIRM brings together experts working across a wide range of disciplines and disease areas."

He says being involved at the grant application review stage is fascinating because he sees firsthand the fruits of CIRM's long investment and has the opportunity to evaluate projects that are now in clinical trials or well on their way toward them.

"Seeing the stem cell field grow to where it is today is a thrilling adventure. I feel privileged to be working with CIRM to help create a better future."

2017 CIRM GAME BALL WINNERS

Every day around the world, lives are on the line. That's why CIRM's mission—and the motivation of our people—is entirely focused on doing everything we can to make a difference to patients' well-being. Leading the way are our CIRM "Game Ball Winners." By living and breathing what we stand for, and the passion and tenacity we aim to bring to our work, these team members have our recognition and respect.

THERAPEUTICS TEAM

For their dogged determination in seeking out high-quality clinical trial projects that were approved by our Board

WHAT THEY DO:
They lead the search for the best clinical stage research projects and do all they can to help them get funded

WHAT DO THEY MOST LIKE ABOUT WORKING AT CIRM:
The rest of the team

DENISE D'ANGEL
HUMAN RESOURCES OFFICER

For completing 100 percent of CIRM team performance reviews on time.

What do you do at CIRM?

Like everyone at CIRM, my job is to help people. I ensure all salaries, benefits and other payments and concerns are taken care of, so our team can focus on their work. I'm the behind-the-scenes support, so patients who need them get the stem cell treatments that transform their lives.

What do you like most about working at CIRM?

The work we do here saves lives. At the very least, it improves them. My goal has always been to do something meaningful, and it's fulfilling knowing I'm part of a team that believes in this, too. It really brings us together and connects us to the people we're helping—at least for a moment, if not for life.

LILIA LEAL
SUPERVISING FINANCE OFFICER, FINANCE TEAM

For her instrumental role in reducing the grant and honoraria payment processing time by 30 days.

What's your role at CIRM?

I make sure we issue our grant payments correctly and pay our bills on time. This means our team always has the resources needed to fulfill what CIRM has set out to do and there's no disruption to our service.

What's your favorite part of the job?

I love what we're about: speeding up how we get more effective stem cell treatments to patients who need them. Everyone is very supportive, smart and caring, and we work well together. I really like coming to work every day.

SHYAM PATEL, Ph.D.
SENIOR SCIENCE OFFICER, PORTFOLIO DEVELOPMENT AND REVIEW

For completing 100 percent of our research grant application reviews on time.

Tell us about your work at CIRM.

I help fund the development of stem cell treatments with the greatest potential to improve the lives of patients. I ensure leading experts in the field review every grant application fairly for scientific and clinical merit.

What's the highlight?

CIRM is a truly collaborative environment, and I love working with our highly dedicated and passionate people. I also love that every day I learn about novel, innovative and disruptive approaches to treating and curing patients with unmet medical needs.

PAUL WEBB, Ph.D.
SENIOR SCIENCE OFFICER AND PROGRAM MANAGER, THERAPEUTICS TEAM

For completing 40 Clinical Advisory Panel (CAP) meetings in less than six months.

How do you help transform lives?

CIRM awards money to scientists and doctors who aim to develop stem cell-based treatments for diseases that have no cure. I work with these scientists and doctors to make this happen more effectively and quickly. My job is to set up conferences with experts—both researchers and patient advocates—who can advise the team on problems they experience as they try to bring stem cell treatments to patients.

Your best daily moment at work?

I like the feeling that comes with helping bring cures to patients.

Every moment counts. Don't stop now.

RYAN WELLS
GRANTS MANAGEMENT SPECIALIST

For money management that keeps CIRM on track to achieve our mission.

What do you do at CIRM?

When scientists apply for and receive a CIRM funding grant for their research projects, I help set up a formal contract for this funding and have it signed by the appropriate people. I also manage paying out the grant award to scientists, review their research budgets and make sure our guidelines for spending grant money are followed.

What do you like most about what you do each day?

CIRM provides an interesting professional challenge. We're always adapting to the ever-changing nature of our award portfolios—whether that's basic research, training projects or clinical trials.

GEOFF LOMAX
SENIOR OFFICER FOR MEDICAL AFFAIRS AND STRATEGIC CENTERS

For getting two new Alpha Stem Cell Clinic Awards approved on time.

What do you do at CIRM?

I coordinate the CIRM Alpha Stem Cell Clinics Network and support other policy-related initiatives.

What do you like most about working at CIRM?

It's a lean, mean treatment-producing machine where everyone has an open-door policy so we can share great ideas.

CHRISTINE BROWN, Ph.D.
CITY OF HOPE NATIONAL MEDICAL CENTER

RESEARCHER

CAR-T CELL THERAPY IS ONE OF THE MOST EXCITING NEW APPROACHES TO TARGET DEADLY CANCERS

City of Hope's Christine Brown, Ph.D., the Heritage Provider Network Professor in Immunotherapy, is using CAR-T cells in Phase 1 of a CIRM-funded clinical trial to target an aggressive brain cancer called malignant glioma. As part of this treatment, a patient's own immune system cells are reengineered to help them fight back against the tumor.

"If it were easy, we'd have cured it by now," she explains.

Of all solid tumors, brain tumors are the hardest to treat, and there are many ways tumors can evade the immune system. In her research, Dr. Brown is looking at different approaches to overcome these challenges, including combining CAR-T cells with other therapies.

"It's one big puzzle about understanding human biology and how we can make new therapies work more effectively."

"We've seen two recent approvals of CAR-T therapies for cancer, where a patient's own immune cells are reengineered—using the tools of gene therapy—to target a patient's individual cancer. This form of gene therapy represents a whole new paradigm in treating cancer. And the early results are changing the way we treat serious tumors.

This experience shows how a single, fundamental breakthrough in science can open up a whole new way of combatting disease."

Testimony of
FDA Commissioner Scott Gottlieb, M.D.
before the U.S. Senate Committee on Health, Education, Labor & Pensions

#PERSISTENT

PATIENT
CALEB SIZEMORE
DIAGNOSED WITH DUCHENNE MUSCULAR DYSTROPHY (DMD)

At just 20 years old, Caleb Sizemore has Duchenne Muscular Dystrophy, a genetic disease that slowly attacks the muscles. Caleb has difficulty standing for long periods, walking and climbing stairs, but he is persistent.

Because the disease also attacks the heart and lungs, many people with DMD don't live beyond their 20s. Caleb is on medication to control scarring on his heart, but the medication cannot undo the damage.

As a patient in the Capricor HOPE trial, Caleb received a transfusion of a cardiac-derived stem cell product into his heart—a therapy being tested for its ability to reverse scarring caused by DMD.

That's a big high five for Caleb, his family and the DMD community.

Capricor, the company running this trial, has now been given permission by the FDA to run another, larger trial for DMD.

"It's an amazing miracle, thanks to CIRM for funding this stem cell treatment."

Learn More:
[Video] Caleb Sizemore: Stem Cell Therapy for Duchenne Muscular Dystrophy

TRANSFORMING THE FUTURE: *financials*

2017 FINANCIAL RECONCILIATION

AWARD ACTIVITY FOR 2017 CALENDAR YEAR

Our strategic plan fiscal projections remain on track. During the year, the uncommitted balance—the amount of money remaining that CIRM has not allocated to a specific project—dropped from $528 million to $270 million. This was the result of $262 million in new awards being issued, offset by $41 million in active award reductions. CIRM currently has $464.5 million under active management on 263 projects and $335 million that will be made available for new awards through mid-2020.

AS OF JANUARY 1, 2017	ROUNDS
• COMMITTED/UNCOMMITTED BALANCE • NUMBER/BALANCE UNDER ACTIVE MANAGEMENT	$2.22B/$528M 255/$406M
2017 ESTIMATED ACTIVITY	
• 88 AWARDS ADDED • AWARDS REDUCTIONS	$302.5M $41.9M
AS OF DECEMBER 31, 2017	
• COMMITTED/UNCOMMITTED BALANCE • NUMBER/BALANCE UNDER ACTIVE MANAGEMENT	$2.48B/$267M 263/$435M
ESTIMATED FUTURE RECOVERY (2018-2019)	$68M
PROJECTED FUNDING AVAILABLE FOR NEW AWARDS	**$335 MILLION**

2018 APPROVED BUDGET

APPROVED BUDGET FOR 2018 CALENDAR YEAR

The type, number and dollar value of budgeted 2018 awards. (Awards are for guidance purposes and are nonbinding.)

The board-approved budget for 2018 calls for $170 million in new investment, which approximates to 30 new awards.

PROGRAM	INVESTMENT
EDUCATION	$0.8M
DISCOVERY	$10M
TRANSLATION	$30M
CLINICAL	$130M
TOTAL	**$170.8 MILLION**

M = MILLION B = BILLION

RISK REVIEW

At CIRM, we're working toward developing cures for diseases responsible for taking more lives than any others—from heart disease and stroke to cancer and diabetes. In our efforts to make a difference, we must face certain risks and overcome challenges. Being aware of what may stand in the way of meeting our goals only makes us more determined to succeed.

CHALLENGES	PERFORMANCE
SUFFICIENT HIGH-QUALITY PROGRAMS FOR 50 NEW CLINICAL TRIALS IN 5 YEARS.	26 NEW TRIALS OVER 2 YEARS.
ADEQUATE INTEREST IN CLINICAL TRIAL PARTICIPATION FROM QUALIFIED APPLICANTS.	RECORD NUMBER OF HIGH-QUALITY APPLICANTS.
CIRM TEAM TURNOVER AND INABILITY TO RECRUIT HIGH-QUALITY REPLACEMENT TALENT.	RETAINING AND RECRUITING TOP TALENT.
INSUFFICIENT INVESTOR INTEREST IN CELL THERAPY.	IN 2017, OVER $389M WAS COMMITTED BY PARTNERS INDEPENDENT OF CIRM FUNDING TO HELP ADVANCE A PROJECT.
PROGRESS STALLED BY REGULATORY ISSUES.	**21ST CENTURY CURES ACT REMOVED OBSTACLES AND LED TO FASTER APPROVAL PATHWAYS. CIRM WAS GRANTED 3 OF THE FIRST 12 FDA RMAT DESIGNATIONS.**

#BRAVE

PATIENT
TOM HOWING
STAGE IV CANCER

In March 2015, Tom Howing was diagnosed with Stage IV cancer throughout his body. After two rounds of surgery, chemotherapy and some promising early results, the cancer returned.

Doctors recommended Tom join the CD47 clinical trial, which uses a combination approach to help a patient's own immune system fight and kill cancer cells.

Phase 1 clinical trials, such as the CD47 trial, are used to determine safety of the treatment—there are still many "unknowns." Even so, Tom's scans and blood tests showed the cancer appeared to be held in check—and that was a win.

Tom has been helping out a local organic farm. "I'm doing surprisingly well," he said. "The treatment is so much less aggressive than chemo."

"I knew I had a battle ahead of me, but I said, 'Let's give it a spin.' Scans show the therapy has dramatically reduced the cancer."

ADRIENNE SHAPIRO
PATIENT ADVOCATE

SPEAKING UP AND STANDING UP

CIRM creates Clinical Advisory Panels for all clinical trials it funds. Each panel includes a CIRM Science Officer, independent scientific experts and a patient representative. Together the team guides and advises researchers to help make each trial a success.

"I'm going to be the last woman in my family to have a child with sickle cell disease."

Adrienne Shapiro is the patient representative on CIRM's Clinical Advisory Panel addressing sickle cell disease—and a force to be reckoned with.

She is committed to addressing the psycho-social needs of patients, knowing how important having strong support is to their recovery.

"The weight of having passed on a genetic disease that hurts my child is enormous," she says. It is what motivates her to be a driving force for a cure and guides her effort as a member of the CIRM panel that brings hope that this disease will disappear.

Adrienne knows she can make a difference and finds satisfaction in speaking for all those with sickle cell disease, certain that her voice—and the voices of others affected by the disease—are heard, loud and clear.

CIRM is funding work by Stanford University's Dr. Matt Porteus that uses the gene-editing tool CRISPR to correct the genetic mutation that causes sickle cell disease. Many scientists consider CRISPR a powerful tool that could help transform our ability to edit out defective genes or insert healthy ones to treat disease.

Learn More:
Patient Stories of Hope

SCIENCE EDUCATION PROGRAMS

At CIRM, we understand that advancing science is a long-term process. That's why we are committed to educating the next generation of stem cell scientists—the talent we need to keep accelerating the field for years and decades to come.

HIGH SCHOOL STUDENTS

CIRM's SPARK Program (Summer Program to Accelerate Regenerative Medicine) is designed to educate high school students about stem cells and give them hands-on experience working in a world-class stem cell research lab. We select students who represent California's diverse population, and particularly those who may not be able to take part in research internships because of financial constraints.

UNDERGRADUATE AND MASTER'S STUDENTS

CIRM's Bridges to Stem Cell Research Awards Program takes educating a new generation of stem cell scientists to the next level. We offer classes and internships at California state schools and community colleges, followed by paid internships at the state's top universities and stem cell labs. Over 1,100 students have completed our Bridges program. More than 50 percent of these students now have full-time lab positions at 20-plus universities and research institutes and 50 biotech and pharmaceutical companies. (These are largely California-based.) A further 30 percent of these students are enrolled in graduate or professional schools.

FURTHER EDUCATION

CIRM also works closely with student programs around the state. Our staff regularly ignites audiences in high schools and colleges with talks and presentations.

In October 2017, Geoff Lomax, CIRM's Senior Officer for Medical Affairs and Strategic Centers, was a featured presenter at University of California, Berkeley's Student Society for Stem Cell Research Annual Symposium. He created a *Jeopardy*-style game to engage students in a debate about stem cell research ethics and policy, turning what could be perceived as a dry topic into a fun, active learning experience.

We also like to connect with audiences in the wider community, giving talks to Rotary clubs and science cafés and joining presentations with aligned organizations like the Foundation Fighting Blindness.

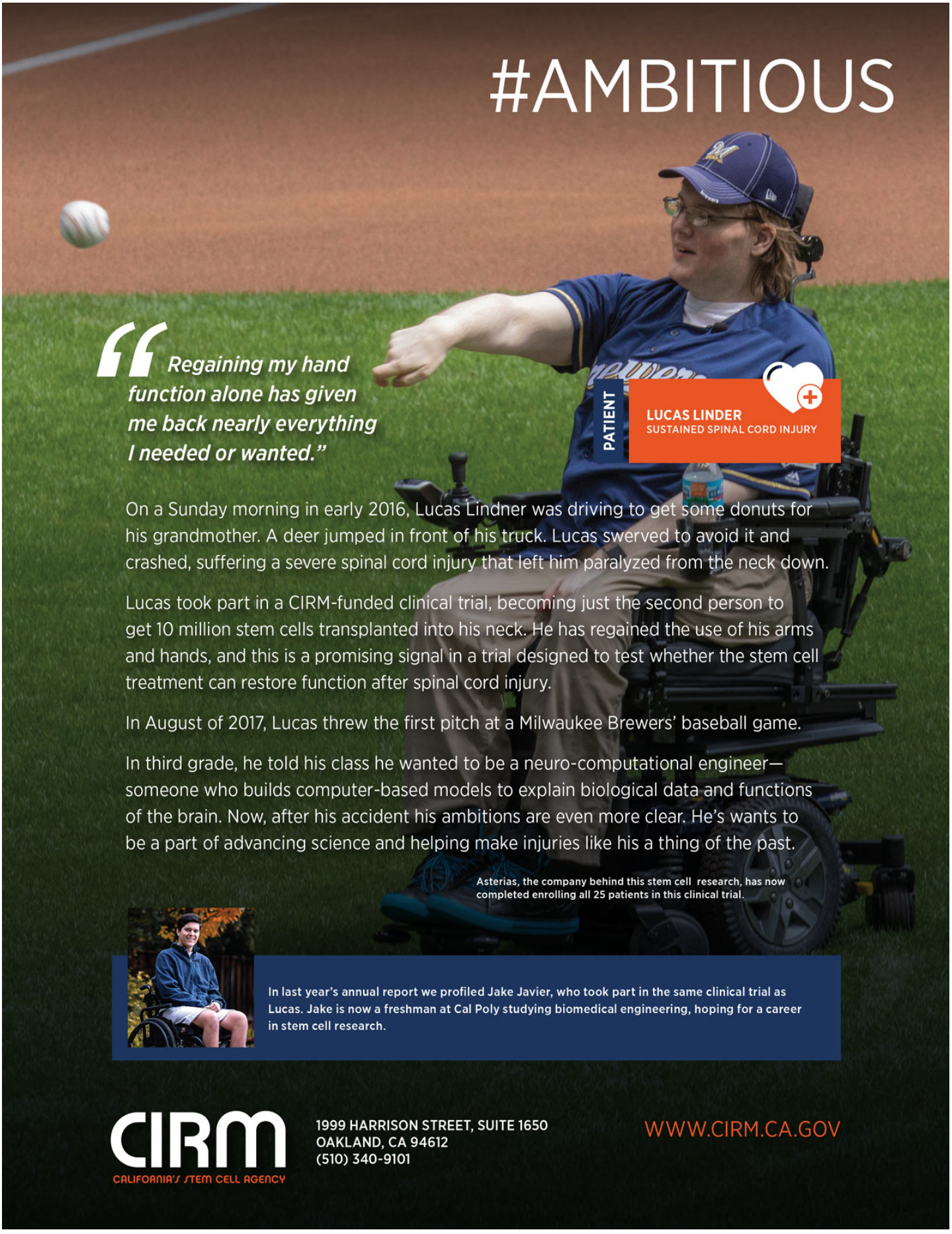
PATIENT ADVOCATE EVENTS

As our biggest supporters, the patient advocate community deserves CIRM's special attention. In dedicating their time, energy and resources to improving the lives of others, it inspires us to continue doing more of the same.

This year we held four events around California: at University of California, San Diego; the Gladstone Institutes in San Francisco; University of California, Davis in Sacramento; and Cedars-Sinai in Los Angeles. Speakers from each host institution discussed their work—including CIRM-funded research—and two members of our own staff showcased research we're supporting and the vital role the patient advocate community plays in this effort.

For us, these patient advocate events are an opportunity to connect with the people who helped create CIRM and to update them on our progress, including how we're investing in therapies we hope will change and save lives.

Learn More:



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